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MULTIPLE DISORDERS—HEALTH POLICY

PMDH1

NICE REQUIREMENTS FOR COST-EFFECTIVENESS EVIDENCE: POTENTIAL SAMPLE SIZE IMPLICATIONS OF GENERATING TRIAL-BASED EVIDENCE

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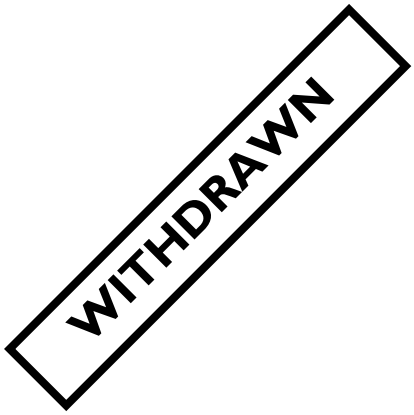
OBJECTIVE: NICE was established in the UK in 1999 to make recommendations about technology adoption based on effectiveness and cost-effectiveness evidence. The ABPI has expressed concern that these requirements will extend product development times and increase development costs. This will depend upon the role NICE expects randomized controlled trials (RCTs) to play in the production of cost-effectiveness evidence, particularly at the time of launch. However, NICE have not yet issued detailed guidance on economic evaluation methodology. Therefore this paper assesses whether a requirement for trial-based economic evidence would increase sample sizes since these are a major determinant of development times and costs. **METHODS:** The University of York Centre for Reviews and Dissemination (CRD) database was used to identify studies, which had collected economic data alongside RCTs, presented cost-effectiveness ratios and reported variances. Publications pertaining to them were retrieved and the data relevant for sample size calculations were extracted. Recently published sample size formulae based on the net health benefits approach were used to calculate the number of subjects which would have been required for testing cost-effectiveness hypotheses. These were compared with those used to evaluate the clinical outcomes in the original trials. **RESULTS:** For each of the studies surveyed, sample sizes would have been significantly greater for performing cost-effectiveness studies than for performing the original clinical trials of which they were a part. However, they are sensitive to the choice of the ratio of acceptable cost-effectiveness used in the calculations. **CONCLUSIONS:** If NICE issues guidance which requires cost-effectiveness evidence based on RCTs to be of the same standard used in the classical approach to clinical evaluations, then the required sample sizes, and hence costs, will most likely be higher than those required for purely clinical evaluation. Larger sample sizes are also likely to extend the period of recruitment and hence development times.

PMDH2



PMDH3

THE USE OF SURROGATE ENDPOINT DATA IN EVALUATING TREATMENT EFFICACY: IMPACT ON DECISION-MAKING AND EXPENDITURE WITHIN THE AUSTRALIAN PHARMACEUTICAL BENEFITS SCHEME

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OBJECTIVE: To investigate the extent to which claims of efficacy in applications for requesting the addition of new drugs to the formulary of the Australian Pharmaceutical Benefits Scheme have been based on surrogate outcome data. **METHODS:** Analyses were drawn from a database of all submissions for new drug/indication pairs considered between 1993 and 1998. Applications were classified according to whether the data presented were based on clincal, intermediate or surrogate endpoints. Drugs utilization and expenditure data were examined